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DEPARTMENT OF PEDIATRICS
DIVISION OF RHEUMATOLOGY AND IMMUNOLOGY

June 28, 2002

Dockets Management Branch
(HFA-305)
Food and Drug Administration
5630 Fishers Lane – Room 1061
Rockville, MD 20652
http://www.fda.gov/dockets/ecomments

Docket Number: 02N-0152

Dear Sir or Madam:

As a pediatric rheumatologist who cares for children and adolescents with juvenile arthritis, systemic lupus erythema and other rheumatologic disorders, I welcome the opportunity to comment on the relationship between the 1998 Pediatric Rule and the Best Pharmaceuticals for Children Act (P.L. 107-109). As a member of the American Academy of Pediatrics (AAP) and on the Section on Pediatric Rheumatology, I know that the AAP has advocated for appropriately tested and labeled medications for infants, children and adolescents for over 40 years. Securing safe and appropriate drugs for use by children has had a long and laborious history. Significant progress toward pediatric drug studies and labeling has been made over the last five years.

A dual approach to obtaining essential pediatric data was instituted in the late 1990's. This approach combines: 1) incentives for voluntary studies of drug safety and dosing by industry (extended in January 2002 in the Best Pharmaceuticals for Children Act [BPCA]); and 2) a regulation requiring pediatric studies of new drugs and some already marketed drugs, known as the Pediatric Rule.

In March 2002 the FDA proposed to suspend the Pediatric Rule. While this proposal was reversed, this action indicates that children are at risk of losing the ground we have fought so hard to secure for them.

The Pediatric Rule ensures that children are no longer a therapeutic afterthought by the pharmaceutical industry. It is an essential and successful tool in ensuring that children have the quality and quantity of drugs they need. All new drugs must be studied for pediatric use at the time a drug comes to market unless the FDA grants a waiver. This makes medications for children a certainty, not an option and puts children on a level playing field with adults for the first time. The illnesses I treat frequently require

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sophisticated combinations of medication. Without the Pediatric Rule I expect the options for approved drugs to administer to my patients will be significantly limited.

I believe that all components of the 1998 Pediatric Rule must be preserved. It is a comprehensive approach to securing pediatric studies. FDA has not yet invoked all the provisions of the Pediatric Rule; however, together they weave a safety net for children to ensure that children have appropriate drugs available for their use.

The following comments and recommendations respond to questions and issues raised in the Federal Register notice soliciting public comments:

- Retiring or relaxing any authorities currently in the Pediatric Rule is inappropriate and would be to the detriment of children. It must always be kept in mind that BPCA is time-limited, voluntary and subject to continuation by the Congress. Those facts speak directly to the need to ensure that the Pediatric Rule remains in place in its entirety.

- Noting again that the BPCA is subject to continuation by Congress and that future reauthorization is uncertain, the Pediatric Rule should mirror the scope of the BPCA and apply to all labeled and potential indications as well as new indications. If a company submits a supplemental indication to the FDA, it invokes the Pediatric Rule. It is important that appropriate pediatric studies be conducted for that new use; and if the current label lacks appropriate pediatric use information (e.g., for neonates) the FDA should also include in their requirement for pediatric studies of the new indication, any pediatric studies that may be needed for the currently labeled or potential indications.

- In determining the process of when pediatric studies are conducted, the FDA should rely on the detailed process for requesting pediatric studies of already marketed drugs and securing labeling that is outlined in the BPCA.

- It is essential that the Pediatric Rule remain in place because it is the only mechanism that ensures that biological products will be studied and available for children. No provision of BPCA applies specifically to biological products since the legislation focuses on drugs covered by the Food, Drug and Cosmetic Act (FDCA) and the vast majority of biologics are covered under the Public Health Service Act. Moreover, some of the most innovative new therapies now and in the future are biological products, which are not covered under BPCA.

- Appropriate formulations are an essential component of providing medications for the pediatric population and especially those patients, patients with complicated and chronic illness, such as those that I treat. It is a requisite for studies in infants and younger children to develop age appropriate formulations, if necessary. Failure to require needed formulations for a specific age population negates the intent of the BPCA and the Pediatric Rule.

- BPCA limits its reference to "recommendation" for formulation changes only to studies completed under public contract. This provision was included to acknowledge

that once a formulation is developed in the study phase, while it may be necessary to manufacture that formulation, it may not always be possible to scale up the formulation for distribution to the general public.

Thank you for your consideration of these comments.

Sincerely,

David Siegel /ds

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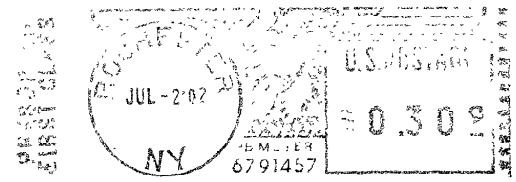

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